Agency for Healthcare Research and Quality
Advancing Excellence in Health Care

NATIONAL GUIDELINE CLEARINGHOUSE

General

Guideline Title

Stem cell transplantation in primary systemic amyloidosis.

Bibliographic Source(s)

Kouroukis CT, Rumble RB. Stem cell transplantation in primary systemic amyloidosis. Toronto (ON): Cancer Care Ontario (CCO); 2012 Mar 29. Various p. (Recommendation report; no. SCT-2). [7 references]

Guideline Status

This is the current release of the guideline.

The RECOMMENDATION REPORT, initially the full original Guideline, over time will expand to contain new information emerging from their reviewing and updating activities.

Please visit the Cancer Care Ontario Web site for details on any new evidence that has emerged and implications to the guidelines.

Recommendations

Major Recommendations

- High-dose chemotherapy (HDCT) and autologous stem cell transplantation (ASCT) is an option for selected patients with primary systemic amyloidosis, preferably within an investigative setting.
- Allogeneic stem cell transplantation (SCT) is not recommended for patients with primary systemic amyloidosis.

Clinical Algorithm(s)

None provided

Scope

Disease/Condition(s)

Primary systemic (amyloid light-chain [AL]) amyloidosis

Guideline Category

Assessment of Therapeutic Effectiveness

Treatment

Clinical Specialty

Hematology

Oncology

Intended Users

Physicians

Guideline Objective(s)

- To evaluate the role of stem cell transplantation (SCT) in the treatment of primary systemic (amyloid light-chain [AL]) amyloidosis
- To review the most current evidence comparing conventional chemotherapy (CT) with high-dose chemotherapy (HDCT) with autologous stem cell transplantation (ASCT)
- To make a series of clinical recommendations to inform clinicians, patients, and other stakeholders of the treatment options available

Target Population

All adult patients with primary (amyloid light-chain [AL]) amyloidosis who are being considered for treatment that includes either bone marrow or stem cell transplantation (SCT)

Interventions and Practices Considered

1. High-dose chemotherapy (HDCT)
2. Autologous stem cell transplantation (ASCT)
3. Allogeneic stem cell transplantation (SCT) (not recommended for patients with primary systemic amyloidosis)

Major Outcomes Considered

- Overall survival
- Complete and partial hematological response
- Treatment-related morbidity and mortality

Methodology

Methods Used to Collect/Select the Evidence

Searches of Electronic Databases

Description of Methods Used to Collect/Select the Evidence

Literature Search Strategy

The MEDLINE (OVID) database (2006 through October [week two] 2010) was systematically searched for evidence on October 21, 2010 using the strategy that appears in Appendix A of the original guideline document. A total of 23 hits were obtained, and after excluding irrelevant papers according to a title and abstract review, three were ordered for full-text review. Of these three, only one met the inclusion criteria and was retained.

Study Selection Criteria

Inclusion Criteria

Articles were selected if they were the following:

1. Systematic reviews (SRs) with or without meta-analysis or clinical practice guidelines (CPGs) if the evidence was obtained with an SR
2. Fully published randomized controlled trials (RCTs) on patients with amyloidosis who received stem cell transplantation (SCT) that reported on survival and/or quality of life (QoL)
3. Fully published non-randomized studies on patients with amyloidosis who received SCT and had an appropriate contemporaneous control group that reported on survival or QoL
4. Reports published in English only

Number of Source Documents

Only one report, a systematic review with meta-analysis, was included.

Methods Used to Assess the Quality and Strength of the Evidence

Expert Consensus (Committee)

Rating Scheme for the Strength of the Evidence

Not applicable

Methods Used to Analyze the Evidence

Review of Published Meta-Analyses

Systematic Review with Evidence Tables

Description of the Methods Used to Analyze the Evidence

Synthesizing the Evidence

While no pooling was planned, it would be considered if data allow.

Assessment of Study Quality

The quality of the included evidence was assessed as follows. For systematic reviews that would be used as the sole evidence base for the recommendations, the Assessment of Multiple Systematic Reviews (AMSTAR) tool would be used to assess quality. For clinical practice guidelines (CPGs), the Appraisal of Guidelines for Research and Evaluation (AGREE) II Instrument would be used but only if adaptation of the recommendations was being considered. Any meta-analysis would be assessed for quality using criteria similar to that used for randomized controlled trials (RCTs), where appropriate. RCTs would be assessed for quality by examining the following seven criteria: the method of randomization, reporting of blinding, the power and sample size calculation, length of follow-up, reporting details of the statistical analysis, reporting on withdrawals to treatment and other losses to follow-up, and reporting on the sources of funding for the research. Comparative, but non-

randomized, evidence would be assessed according to a full reporting of the patient selection criteria, the interventions each patient received, and of all relevant outcomes.

Methods Used to Formulate the Recommendations

Expert Consensus

Description of Methods Used to Formulate the Recommendations

Not stated

Rating Scheme for the Strength of the Recommendations

Not applicable

Cost Analysis

A formal cost analysis was not performed and published cost analyses were not reviewed.

Method of Guideline Validation

Not stated

Description of Method of Guideline Validation

Not applicable

Evidence Supporting the Recommendations

Type of Evidence Supporting the Recommendations

The recommendations are supported by a systematic review with meta-analysis.

Benefits/Harms of Implementing the Guideline Recommendations

Potential Benefits

Appropriate recommendation regarding stem cell transplantation (SCT) in patients with primary systemic amyloidosis

Potential Harms

- A randomized controlled trial (RCT) found treatment with autologous stem cell transplantation (ASCT) to be associated with a significant increase in treatment-related mortality.
- The ASCT treatment-related morbidity included infection, neutropenia, mucositis, adverse gastrointestinal effects, central nervous system effects (including seizures), acute renal failure, and bacterial sepsis syndrome.

Qualifying Statements

Qualifying Statements

- The patient selection process and the ultimate decision to perform a stem cell transplantation (SCT) should take into account not only disease-related characteristics, but also comorbidities and patient preferences.
- Care has been taken in the preparation of the information contained in this report. Nonetheless, any person seeking to apply or consult the report is expected to use independent medical judgment in the context of individual clinical circumstances or seek out the supervision of a qualified clinician. Cancer Care Ontario makes no representation or guarantees of any kind whatsoever regarding the report content or use or application and disclaims any responsibility for its application or use in any way.

Implementation of the Guideline

Description of Implementation Strategy

An implementation strategy was not provided.

Implementation Tools

Quick Reference Guides/Physician Guides

For information about availability, see the *Availability of Companion Documents* and *Patient Resources* fields below.

Institute of Medicine (IOM) National Healthcare Quality Report Categories

IOM Care Need

Getting Better

Living with Illness

IOM Domain

Effectiveness

Identifying Information and Availability

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Adaptation

Not applicable: The guideline was not adapted from another source.

Date Released

2012 Mar 29

Guideline Developer(s)

Program in Evidence-based Care - State/Local Government Agency [Non-U.S.]

Guideline Developer Comment

The Program in Evidence-based Care (PEBC) is a Province of Ontario initiative sponsored by Cancer Care Ontario and the Ontario Ministry of Health and Long-Term Care.

Source(s) of Funding

The Program in Evidence-based Care (PEBC) is a provincial initiative of Cancer Care Ontario supported by the Ontario Ministry of Health and Long-Term Care through Cancer Care Ontario. All work produced by the PEBC is editorially independent from its funding source.

Guideline Committee

Hematology Disease Site Group

Composition of Group That Authored the Guideline

For a current list of past and present members, please see the Cancer Care Ontario Web site .

Financial Disclosures/Conflicts of Interest

The authors of this recommendation report disclosed potential conflicts of interest relating to the topic of this special advice report and declared there were none.

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Guideline Availability

Electronic copies: Available in Portable Document Format (PDF) from the Cancer Care Ontario Web site .

Availability of Companion Documents

The following are available:

- Stem cell transplantation in primary systemic amyloidosis. Summary. Toronto (ON): Cancer Care Ontario; 2012 Mar 29. 4 p. Electronic copies: Available in Portable Document Format (PDF) from the Cancer Care Ontario (CCO) Web site .
- Program in evidence-based care handbook. Toronto (ON): Cancer Care Ontario (CCO); 2012. 14 p. Available in Portable Document Format (PDF) from the CCO Web site .

Patient Resources

None available

NGC Status

This summary was completed by ECRI Institute on September 6, 2013.

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